



Particular Aspects of Trials in *Medical Rehabilitation*

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Purpose of Clinical Trials

Promote new diagnostic & therapeutic approaches

**Modify and optimize current therapies;
integrate therapies**

Adapt existing therapies to novel patient populations

Challenge assumptions of current practice

Safety and efficacy

Explore potential secondary effects

Longitudinal follow-up

**Evidence-based medicine to drive health-care
management and policy**



Biological Plausibility or Mechanistic Hypothesis

Reducing acute pathophysiology, collateral damage, or secondary complications

Promoting recovery, regeneration, adaptation, or plasticity

Psychosocial benefits

Etc.



Justification for Approach

Preliminary data from anecdotal clinical reports or pilot studies

Animal studies

Therapeutic benefit in related conditions

Correlative evidence (e.g., epidemiological data)



Appropriate Outcome Measures

Appropriate to level of intervention

**Measure across domains of pathophysiology,
impairment, function or disability**

Clinically and ecologically relevant goals

**Test battery versus single measure
(improved sensitivity and validity)**



Appropriate Outcome Measures

(continued)

**Potential for Change in status over time
(recovery, improvement)**

**Outcome measures not overly
burdensome to subjects**

Data analysis techniques

Standards for a “successful” trial



Other Issues in Design of Clinical Trials

**Justification of dosage and timing
of treatment**

Control and treatment groups:

**Placebo, sham, current standard of care,
or other defined procedure**

Dose-response, cross-over design

Timing of outcome measures



Other Issues in Design of Clinical Trials (continued)

Target Patient Population:

Based on pathophysiology, impairment, functional deficit, disability?

Patient Recruitment:

Access to sufficient numbers

Remuneration

Support and attrition



Other Issues in Design of Clinical Trials (continued)

Human subjects issues:

Risk-benefit

Informed consent

Conflict-of-interest

Confidentiality

Clinical networking

Oversight

Coordination & standardization across sites



Special issues for Clinical Trials in Medical Rehabilitation

**Relatively new research field;
lack of research tradition?**

**But long tradition of treatment strategies
Allied fields have solid research tradition
(e.g., physiology, neuroscience)**

**Clinicians lack research training,
especially in clinical trials?**



Special issues for Clinical Trials in Medical Rehabilitation

Lack of evidence-based studies to justify current approaches

Acute need to justify rehabilitation approaches in current managed care environment

Necessity for coordinating multi-disciplinary teams



Special issues for Clinical Trials in Medical Rehabilitation

**Variability of conditions treated;
Need for individualized treatments**

**Treating “function” and “disability” –
further removed from “biological” mechanisms**

**Institutional support:
Clinical Departments
Professional Organizations
Funding agencies**



Themes

[Minimizing acute pathology and reducing collateral damage – NINDS>NCMRR]

Optimizing timing, intensity, and specificity of therapeutic interventions

Adapting pharmacological treatments for use in disabling conditions

(e.g., pain, spasticity, depression, cognitive deficits, memory)



Themes

Promoting neuroplasticity and recovery through activity-driven processes

Exercise to prevent deconditioning/ atrophy, and to promote overall health

Electrophysiological and Neuroprosthetic approaches to supplement function

Preventing reoccurrence and minimizing secondary complications



Themes

Social and psychological support; health promotion; minimizing social barriers;

Improved prioritization of limited health care resources

Replacing function and promoting adaptation

Behavioral modification



Future Opportunities

Molecular, cellular and bioengineering approaches to promote regeneration and restore function

Optimizing therapies for patient conditions and goals

Monitoring progress and outcomes

Adapting adult therapies to children



Future Opportunities

**Telemedicine and home-based therapies;
rural environments**

**Gender and ethnic/racial differences in
recovery processes, health access, support**

Alternative and complementary medicine

**Developing ecological appropriate
interventions for home and community
to support compliance**



NIH Support of Clinical Trials

Support of investigator-initiated clinical trials

Workshops and courses

Training and career development in clinical trials

Clinical Trial planning grants

Requests for applications (RFAs):

certain targeted conditions or approaches

Clinical networks

(recruit patients, standardize therapies)